Submission to the Patented Medicine Prices Review Board
Consultation on three proposed amendments to the new PMPRB Guidelines related to the decision to delay the coming-into-force date of the Regulations Amending the Patented Medicines Regulations from July 1, 2021 to January 1, 2022

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Information Sources

This submission follows input Cystic Fibrosis Canada provided to the PMPRB in June 2021, August and February 2020 on the draft Guidelines, as well as on proposed reforms, provided in February 2018 and June 2017, and input in October 2016 regarding Health Canada's PMPRB Guidelines Modernization Discussion Paper.

In addition, Cystic Fibrosis Canada has had several communications with the PMPRB. We have provided correspondence and participated in the PMPRB's stakeholder briefing sessions. This submission draws on our recommendations from all of these sources, as well as others.

About Cystic Fibrosis

Cystic fibrosis is the most common fatal genetic disease affecting children and young adults in Canada. There is no cure. Cystic fibrosis causes various effects on the body, but mainly affects the digestive system and lungs. The degree of cystic fibrosis severity differs from person to person; however, the persistence and ongoing infection in the lungs, with progressive loss of lung function will eventually lead to death in most people with cystic fibrosis. Respiratory failure causes eighty-five percent of cystic fibrosis fatalities.

Other consequences of having cystic fibrosis include malnutrition and very low BMI, and cystic fibrosis-related comorbidities like cystic fibrosis-related diabetes (CFRD) and cystic fibrosis-related liver disease.

Cystic fibrosis is a complex disease caused by mutations in the gene for the Cystic Fibrosis Trans-membrane Conductance Regulator (CFTR). There are over 2,090 known mutations. Cystic fibrosis has a tremendous impact on the people who live with it, their loved ones, and on society. Every week in Canada, two people are diagnosed with cystic fibrosis, one of them through newborn screening. Every week in Canada, one person with cystic fibrosis will die.

About Cystic Fibrosis Canada

Cystic Fibrosis Canada has dramatically changed the cystic fibrosis story. We have advanced research and care that has more than doubled life expectancy. Since being founded by parents in 1960, Cystic Fibrosis Canada has grown into a leading organization with a central role engaging people living with cystic fibrosis, parents and caregivers, volunteers, researchers and healthcare professionals, government, and donors. We work together to change lives through treatments, research, information, and support. Despite our remarkable progress half of the people with cystic fibrosis who died over the past three years were younger than 34.

We work closely with our patient community to advocate to improve their health and well-being. In 2020, Cystic Fibrosis Canada's National Advocacy Network consisted of 250 well-trained advocates and a basket of tools to help them in their efforts. We have been able to help the cystic fibrosis community by amplifying their voices through coordinated efforts that have addressed both national and regional priorities.

Cystic Fibrosis Canada's contributions have led to significant improvements in care and quality of life for people living with cystic fibrosis. As a result, Canada has one of the highest median ages of survival in the world.

Background

The Patented Medicine Prices Review Board (PMPRB) has invited stakeholders to comment on three proposed amendments to the new PMPRB Guidelines related to the decision to delay the coming-into-force date of the Regulations Amending the Patented Medicines Regulations a further six months, from July 1, 2021 to January 1, 2022. The PMPRB proposed to change the definition of Gap medicines, the references to the comparator countries and the international price tests for Grandfathered medicines and their line extensions.[1]

Proposed Amendments to the July 1, 2021 Guidelines:

1. **Definition of Gap Medicines**
   The definition of Gap medicines under the new Guidelines applies to medicines for which a DIN was assigned on or after August 21, 2019 and prior to July 1, 2021 and first sold in Canada prior to July 1, 2021. The PMPRB is proposing to extend the date on which the DIN was assigned and the date of first sale to the new coming-into-force date of January 1, 2022

2. **Comparator Countries**
   The comparator countries used under the new Guidelines are currently referred to as the "PMPRB11". The PMRPB is proposing to refer to the comparator countries more by reference to the Schedule set out in the Regulations as the "Schedule Countries".

3. **International Price Tests for Grandfathered Medicines and their Line Extensions**
   Under the new Guidelines, the maximum list price ("MLP") for Grandfathered and Line extensions is set by the lower of (1) the highest international price ("HIP") for the PMPRB11 countries for which the patentee has provided information; or (2) the medicine's ceiling (e.g. the "NEAP") under the Guidelines as they were prior to the issuance of these guidelines.

The PMPRB is proposing that the MLP for Grandfathered and their Line Extensions be set by the lower of (1) the MIP for the Schedule Countries for which the patentee has provided information for the reporting period ending June 30, 2021 under the Regulations that are currently in effect (SOR/2008-70, s.6); or (2) the medicine's ceiling (e.g. the "NEAP") under the Guidelines as they were prior to the issuance of these Guidelines. For Grandfathered and their Line Extension medicines first filed with the PMPRB for the reporting period(s) ending in

[1] https://www.canada.ca/en/patented-medicine-prices-review/services/consultations/notice-comment-references-comparator-countries.html August 8, 2021.

December 31, 2021, or later, the MLP is set by the HIP for the Schedule Countries set out in the Regulations for which the patentee has provided information.

Cystic Fibrosis Canada's Response

Cystic Fibrosis Canada supports efforts to lower the costs of prescription drugs for Canadians. We believe that this must not inhibit access to new drugs but can and must be done in a way that ensures timely access by Canadians to new medicines, especially innovative and precision medicines.

Cystic Fibrosis Canada is disappointed that the concerns of patients raised in the initial consultation process in 2017 appear to have been largely ignored by the PMPRB. The PMPRB does not seem to have seriously weighed the concerns of patients and patient organizations like Cystic Fibrosis Canada and as a result, the consultation processes that have been offered to stakeholders appear to us to have been primarily symbolic.

Throughout these ongoing communications and consultations, we learned that feeling unheard and under-valued is a common thread among many patients and patient groups when it comes to engaging with, and being consulted by, the Patented Medicines Prices Review Board.

Patients and patient groups are primary stakeholders in healthcare, particularly with respect to access to medicines. As primary stakeholders, we worked hard to demonstrate the opportunities and challenges we saw with respect to the implementation of the PMPRB changes. Throughout the consultation periods, we stressed, again and again, that we agreed with the goal of lowering drug prices and the changes to the comparator countries, but we had concerns that the additional pharmacoeconomic elements being proposed would negatively impact access. The PMPRB told us, again and again, that our fears were unfounded. Little to no changes were made to the guidelines to address our concerns.

Specific Comments on the Proposed Amendments to the July 1, 2021 Guidelines:

1. Definition of Gap Medicines

We agree with the change in the definition of Gap medicines under the new Guidelines.

2. Comparator Countries

Referring to the comparator countries by reference to the Schedule set out in the Regulations as the "Schedule Countries" is reasonable.

We believe that the PMPRB's failure to implement the now called "Schedule Countries" sooner, as many stakeholders suggested, has sadly resulted in a lost opportunity that could have saved millions of dollars, money that could have been spent on getting medicines to people rather than delaying access by creating a prolonged environment of uncertainty for drug manufacturers by drawing out the implementation process.

3. International Price Tests for Grandfathered Medicines and their Line Extensions

While Cystic Fibrosis Canada agrees that changing the basket of comparator countries will have the desired effect of lowering the costs of drugs in Canada we believe that implementing additional measures to further reduce prices will only serve to make Canada an outlier with respect to its OECD counterparts, making it an unfavourable market for the pharmaceutical industry.

Cystic Fibrosis Canada appreciates and supports efforts by the PMPRB to manage excessive drug prices but believe that the pricing pendulum has swung too far, too fast, with no serious consideration of the information gathered during the initial consultation on what these changes will mean to patients. The proposed amendments have already created a chilling regulatory, review and reimbursement environment, one in which manufacturers are questioning whether or not to launch their products in Canada.

With respect to the proposed process to determine maximum list price ("MLP") for Grandfathered and Line extensions we feel that the PMPRB should focus on implementing only the Schedule Countries and halt the implementation of any new economic criteria until an independent third party can evaluate their impact.

Cystic Fibrosis Canada's Recommendations

Since the very beginning of the consultations on the regulatory changes and the guidelines, Cystic Fibrosis Canada, along with many other stakeholders, has provide consistent feedback that has yet to be addressed by the PMPRB.

Again, our recommendations are:

RECOMMENDATION 1:

Cystic Fibrosis Canada urges the PMPRB to implement only the changes to the Schedule Countries and to put on hold any guidelines aimed at further reducing prices until the impact of the new economic criteria have been thoroughly evaluated by an independent third party.

RECOMMENDATION 2:

Cystic Fibrosis Canada recommends that an independent third party evaluate the impact of the new economic criteria on the availability of medicines in Canada specifically to inform any decision on whether and how to implement the use of the new economic criteria for innovative, precision and other high-cost medicines.

RECOMMENDATION 3:

That the Federal Government require that PMPRB, along with other appropriate agencies, immediately establish a formal mechanism for meaningfully and continuously engaging patient representatives in its decision-making and processes to ensure patient voice, choice and representation.

While the PMPRB has proposed the GMEP model of evaluation, we do not believe that the PMPRB is well suited to evaluate itself. In its own words, the PMPRB notes that through the GMEP, it will seek to monitor and evaluate trends in the pharmaceutical market that may be impacting patentees, as well as the consumers, patients, and payers that it is mandated to protect. Given its record on patient engagement and consultation, Cystic Fibrosis Canada has serious concerns about the approach and conduct of the PMPRB and does not believe it is the best interest of Canadians to have this body monitor and evaluate the impact of its own regulatory and guideline changes.

Should you have any questions, please contact:

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